

Key Questions for Guiding Clinical Research and Developing Evidence-based Guidelines

Topic 1: Weight-based dosing strategies

P: Adults or children with hemophilia receiving factor replacement treatment

I: Factor dosing based on ideal body weight or adjusted body weight

C: Factor dosing based on actual body weight

O: Pharmacokinetic measurements of effectiveness, total factor use, long-term joint outcomes (arthropathy), cardiovascular events, cost-effectiveness

KQ1: What is the comparative effectiveness and comparative cost-effectiveness of factor dosing based on ideal body weight or adjusted body weight?

KQ2: Does the comparative effectiveness of factor dosing based on ideal body weight or adjusted body weight vary based on:

- a. Patient characteristics (age, hemophilia type, presence of inhibitors)
- b. Prophylactic use vs treatment use
- c. Type of factor replacement

Topic 2: Use of ultrasound

P: Adults or children with hemophilia

I: Use of ultrasound for diagnosis of acute joint bleeding or routine assessment of joint health

C: Usual care (clinical assessment, goniometry, patient questionnaires), magnetic resonance imaging

O: Long-term joint outcomes (arthropathy), change in management, total factor use, diagnostic accuracy or time to diagnosis, cost-effectiveness

KQ1: What is the comparative effectiveness and comparative cost-effectiveness of ultrasound to diagnose acute joint bleeding or to periodically assess joint health?

KQ2: Does the effectiveness of ultrasound to assess joint bleeding or joint health vary based on:

- a. Patient characteristics
- b. Presence of severe degenerative joint changes
- c. History of prior joint bleeding
- d. Operator experience

KQ3: Does ultrasound improve diagnostic accuracy or time to diagnosis for acute hemarthrosis?

KQ4: Does ultrasound improve diagnostic accuracy or time to diagnosis of chronic arthropathy?

Topic 3: Home care services

P: Adults or children with hemophilia at increased risk of bleeding due to surgery or other situational factors

I: Continuous or episodic home nursing care or episodic multidisciplinary home care team visits

C: Usual care

O: Joint bleeding, change in management, total factor use, hospital admission or readmission

KQ1: What is the effectiveness of continuous or episodic home nursing care for patients with hemophilia at increased risk of bleeding?

KQ2: What is the effectiveness of episodic multidisciplinary home care team visits for patients with hemophilia at increased risk of bleeding?

Contextual Question: What strategies or pathways have been described for utilization management of factor replacement for patients with hemophilia?